Novartis International AG
CH-4002 Basel
Switzerland

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PRESS RELEASE

Novartis receives European Commission approval for Itvisma® for spinal muscular atrophy (SMA)

- *First gene replacement therapy in the EU for broad population with SMA, including children two years and older, teens and adults*
- *Fixed, one-time dose designed to replace the faulty SMN1 gene, providing a distinct option from ongoing dosing approaches*
- *Novartis gene replacement therapies now eligible in the EU for people with SMA across all age groups*

Basel, July 2, 2026 – Novartis today announced that the European Commission (EC) has approved Itvisma® (onasemnogene abeparvovec) for the treatment of children two years and older, teens and adults living with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the survival motor neuron 1 (*SMN1*) gene.

With this approval, Itvisma becomes the first and only gene replacement therapy currently approved for this broad SMA population in the European Union.

Itvisma is uniquely designed to address the genetic root cause of SMA with a one-time fixed dose that does not need to be adjusted for age or body weight.[1] By replacing the *SMN1* gene, Itvisma can improve motor function, offering a one-time treatment option distinct from ongoing dosing approaches associated with other available therapies for this population.[1]

“European approval is an important milestone for the SMA community. Beyond the scientific achievement, it brings the prospect of a new treatment option closer to people and families who are looking for choices that reflect their individual needs and circumstances. We welcome today’s decision and hope it translates into timely and equitable access across Europe,” said Nicole Gusset, CEO of SMA Europe.

“Maintaining or improving motor function can make a meaningful difference for older children, teens and adults living with SMA,” said Professor Jana Haberlová, Head of Neuromuscular Centre at the Department of Paediatric Neurology, Motol and Homolka University Hospital, Prague, Czech Republic. “The approval of Itvisma in Europe is an important advance because it brings a new gene replacement therapy option to a broader patient population and gives clinicians an additional way to support patients across the course of the disease.”

The approval is based on data from the registrational STEER study, and supportive Phase IIIb STRENGTH and Phase I/II STRONG studies.[1,2,3] In STEER, Itvisma demonstrated a statistically significant 2.39-point improvement in the Hammersmith Functional Motor Scale (HFMSE) with effects sustained over 52 weeks of follow-up.[1] The STEER and STRENGTH studies also showed clinically meaningful benefit for treatment-naïve and pre-treated patients.[1,2]

“This approval marks a major milestone for people living with SMA,” said Patrick Horber, MD, President, International, Novartis. “With Itvisma, we are going further to expand access to a one-time gene replacement therapy for older children, teens and adults – potentially addressing long-standing unmet needs for patients. Together with Zolgensma, we can now offer gene replacement therapy options across different stages of SMA in Europe, from newborns to adults.”

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About SMA
Spinal muscular atrophy (SMA) is a rare, genetic neuromuscular disease caused by a mutated or missing *SMN1* gene.[4,5] The *SMN1* gene is responsible for producing most of the SMN protein a body needs for muscle function, including breathing, swallowing and basic movement.[5] Without it, motor neurons are irreversibly lost, leading to progressive, debilitating muscle weakness.[5] A second gene, the *SMN2* gene, produces a small fraction (~10%) of functional SMN protein compared with the *SMN1* gene.[6] Individuals with more copies of the *SMN2* gene generally have a less severe form of SMA than those with fewer copies.[6] SMA has an estimated global prevalence of around 1 to 2 per 100,000 people, with an incidence of roughly 1 in 10,000 live births.[7]

About Itvisma® (onasemnogene abeparvovec)
Itvisma, an adeno-associated virus 9 (AAV9)-based gene therapy, is uniquely designed to address the genetic root cause of SMA by providing a functional copy of the human *SMN1* gene to improve motor function through sustained SMN protein expression with a single, one-time intrathecal injection. The most common side effects with Itvisma include upper respiratory tract infection, pyrexia, vomiting, headache and increased hepatic enzymes.[1,2,3] Results from the STEER and STRENGTH studies were published in *Nature Medicine*.[1,2]

Novartis has an exclusive, worldwide license with Nationwide Children's Hospital to both the intravenous and intrathecal delivery of AAV9 gene replacement therapy for the treatment of all types of SMA; an exclusive, worldwide license from REGENXBIO for any recombinant AAV vector in its intellectual property portfolio for the in vivo gene replacement therapy treatment of SMA in humans; an exclusive, worldwide licensing agreement with Généthon for in vivo delivery of AAV9 vector into the central nervous system for the treatment of SMA.

Novartis in neuroscience
Neurological diseases are deeply personal, affecting people of any age, from newborns to seniors, often striking in the prime of life. At Novartis, we're doubling down on our commitment to neurology, expanding our legacy of innovation in SMA and multiple sclerosis (MS) to work in neuroimmunology, neurodegeneration, and neuromuscular diseases. Our goal is to protect people’s health across their lifespan, developing more treatment options that lead to better outcomes.

Disclaimer
This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as “potential,” “can,” “will,” “plan,” “may,” “could,” “would,” “expect,” “believe,” “committed,” “commitment,” “pipeline,” “launch,” “potentially,” “step forward,” “goal,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for Itvisma, or regarding potential future revenues from Itvisma. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that Itvisma will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that Itvisma will be commercially successful in the future. In particular, our expectations regarding Itvisma could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG’s current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

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About Novartis
Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach more than 300 million people worldwide.

Reimagine medicine with us: Visit us at **https://www.novartis.com** and connect with us on **LinkedIn**, **Facebook**, **X/Twitter** and **Instagram**.

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Novartis Media Relations
E-mail: media.relations@novartis.com

Novartis Investor Relations
Central investor relations line: +41 61 324 7944
E-mail: investor.relations@novartis.com